

## ATTACHMENT NO. 3

### Non-Technical Abstract

The NT-501 encapsulated cell implant is a drug delivery system, engineered to deliver human ciliary neurotrophic growth factor (CNTF) to the eye after implantation. The intent of this CNTF delivery is to arrest the progressive loss of photoreceptors which is characteristic of retinitis pigmentosa (RP) and related conditions.

Many studies have demonstrated the promise of growth factors as therapeutics for RP in animals. Among these growth factors, CNTF is reported to be the most effective in reducing retinal degeneration. Unfortunately, systemic administration of these agents for treatment of RP has proven to be impractical. In order to circumvent these CNTF delivery problems, the NT-501 unit has been developed.

Each NT-501 unit consists of a sealed semipermeable hollow fiber membrane (HFM) capsule surrounding six strands of polyethylene terephthalate (PET) yarn which have been loaded with CNTF-secreting NTC-201 cells (derived from a human retinal pigment cell line). The ends of each capsule are sealed with glue after loading with the cells. This secures the cells within the NT-501 unit. A titanium loop facilitates placement and retrieval of the implanted device, and is attached to one end of the device. The HFM allow for the outward diffusion of CNTF and other cellular products and the inward diffusion of nutrients necessary to support cell survival. After implantation CNTF is released into the fluids of the eye from the cells. In addition, the cells within the device are protected from the host's immune system. The NT-501 is manufactured to be sterile and retrievable. The current device is about 1.1 cm in length (inclusive of the titanium loop) and will be placed well outside the visual axis in the human eye.

The NT-501 releases CNTF but not the cells contained therein. That is, the NT-501 is a "mini-factory" for CNTF delivery *in situ* but poses no direct patient exposure to the cells it contains or their genetic components.